CIRM Funded Clinical Trials

A Clinical Trial to Evaluate the Safety and Efficacy of RP-L201 in Subjects With Leukocyte Adhesion Deficiency-I

Disease Area:	Leukocyte Adhesion Deficiency
Investigator:	Kinnari Patel
Institution:	Rocket Pharmaceuticals, Inc.
CIRM Grant:	CLIN2-11480 (Pre-Active)
Award Value:	$6,567,085
Trial Sponsor:	Rocket Pharmaceuticals, Inc.
Trial Stage:	Phase 1/2
Trial Status:	Recruiting
Targeted Enrollment:	9
ClinicalTrials.gov ID:	NCT03812263

Kinnari Patel

Details:
Leukocyte Adhesion Deficiency-I (LAD-I) is a rare pediatric disease caused by a mutation in a specific gene that affects the body's ability to combat infections. As a result, infants with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Those that survive infancy experience recurrent severe infections, with mortality rates for severe LAD-I at 60-75% prior to the age of two and survival very rare beyond the age of five.

Rocket Pharmaceuticals, Inc. will test a treatment that uses a patient's own blood stem cells and inserts a functional version of the gene. These modified stem cells are then reintroduced back into the patient that would give rise to functional immune cells, thereby enabling the body to combat infections.

Design:
This is a pediatric non-randomized open-label Phase I/II clinical trial.

Goal:
Safety and efficacy

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